DEPARTMENT OF HEALTH & HUMAN SERVICES Public Health Service

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TO: Director, Office of Rare Diseases

FROM: Senior Investigator, RNA Regulation Section, LCMB, NIA

SUBJECT: ORD Financial Support for Scientific Conference entitled **"RNA Regulatory Mechanisms Implicated in Human Diseases Both Rare and Common"**

The conference entitled **"RNA Regulatory Mechanisms Implicated in Human Diseases Both Rare and Common"** was held in Asheville, NC, October 12th-16th, 2008. The meeting was organized by Jack Keene (Duke Univ. Med. Ctr.), Perry Blackshear (NIEHS), Gary Brewer (UMDNJ, R.W. Johnson Med. School), Myriam Gorospe (NIA), and David Port (Univ. Colorado HSC). Paul Anderson (Harvard Medical School) was an ad hoc member of the committee. This meeting is the third in a series, the first two of which were held in Florence (Italy) in 2003 and in Arolla (Switzerland) in 2005. The 2008 meeting took place in Asheville, North Carolina at the Crowne Plaza Hotel, a venue that was proximal to the institutions of several organizers and many of the planned attendees.

The topic of RNA stability has broad disease implications including cancer, diabetes, inflammatory, aging, neurodegenerative, and infectious diseases, and in fact, most other human diseases. Thus, the committee invited speakers who represented the most outstanding researchers in the field of RNA stability and translation, whose work spans many human models of physiology and pathology as well as other mammalian and lower eukaryotic model systems.

The meeting was divided into 6 sessions, each consisting of 8 presentations:

1. **General RNA Turnover**
2. **ARE and NMD Decay**
3. **Decay and Translation**
4. **RBPs and miRNAs**
5. **Biological Responses**
6. **Disease Implications**

The sessions covered various molecular mechanisms and cellular interactions that govern RNA stability in many disease-relevant systems, as discussed in both oral and poster presentations. Data were presented on RNA-binding proteins that affect RNA stability and/or translational control in paraneoplastic encephalomyelitis disorders and fragile X syndrome. Evidence was also shown for a role of RNA-binding proteins in the adult-onset neurodegenerative disease amyotrophic lateral sclerosis (ALS). The dysregulation of mRNA stability was found to contribute to rare pancreatic diseases, including pancreatic cancer. Proteins involved in rare hereditary cancer syndromes (such as von Hippel Lindau, pVHL), were demonstrated to have a component of altered mRNA turnover/translation. Work was also presented to support the notion that several immune disorders, including autoimmune diseases

such as lupus, Sjogren's syndrome and polymyositis/ dermatomyositis, involve the formation of autoantibodies reactive with RNA-binding proteins that affect RNA stability. Infectious organisms (e.g. trypanosomes) and viruses (e.g. herpes) gain advantages by using RNA stability to block host functions and/or to replicate themselves, as illustrated in several presentations. Many of these diseases, whether rare or common, involve the functioning of microRNAs and other small regulatory RNAs that interact with messenger RNAs and RNA-binding proteins. Numerous publications will undoubtedly result from these studies in the coming months. This meeting examined the implication of numerous ribonucleoprotein components that regulate RNA stability and translation across many disease types, while considering their implications for etiology, diagnosis and therapy.

The next conference in this series is planned for 2011 in Montreal (Canada). The 2001 Organizing Committee includes I.E. Gallouzi, J. Wilusz, and J.D. Keene. Finally, the committee intends to publish a summary of the meeting similar to one in EMBO Reports 7, 143-148 (2006) following the meeting in 2005. A copy of the published summary will be forwarded to the Office of Rare Diseases.

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